Assessing the Impact of Biomedical Research: Examples of Approaches from the National Cancer Institute (NCI)

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Challenges

- **Time Lags**
  - From basic discovery to translation into interventions
  - From application of new interventions to changes in health statistics

- No single definitive approach to assessing impact

- Linkage & attribution to funding sources
Converging Approaches

- **JCO Advances**: Expert-identified Journal of Clinical Oncology (JCO) clinical scientific advances
- **Biomarkers**: Breast cancer biomarkers in expert-curated Thomson Reuters Integrity℠ database
- **Patent Analysis**: Patents and associated publications underlying FDA-approved drugs – FDA Orange Book
- **NCCN Guidelines**: For cancer treatment, screening & detection, and supportive care - The National Comprehensive Cancer Network® (NCCN®) Guidelines
# Approaches & Relevance to Components of the Research Process

<table>
<thead>
<tr>
<th></th>
<th>Research Findings</th>
<th>Technology and Clinical Development</th>
<th>Approved Products</th>
<th>Clinical Practice</th>
<th>Societal Benefits</th>
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<td><strong>JCO Advances</strong></td>
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<td><strong>NCCN Guidelines</strong></td>
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*National Cancer Institute*
What is the role of NCI support in expert-identified scientific advances?

- **Source**: Journal of Clinical Oncology (JCO) Clinical Cancer Advances Series
- **Key data**: Expert panel-identified “major advances” and “notable research” from JCO “Clinical Cancer Advances” series (2005-2012)
- **Basis**: cited journal articles, conference presentations, FDA press releases and underlying clinical trials
- **Linkage to NCI**: funding acknowledgements, intramural author affiliations, clinical trial sponsors
Collect and analyze acknowledgements; check list of authors for NCI intramural

Get presentation/watch video

Analyze acknowledgments; Check list of authors for NCI intramural

Find underlying trial(s) in clinicaltrials.gov,

Analyze acknowledgments/authors in journal articles reporting results of trials

Non-NCI acknowledgement

NCI acknowledgement

Extract award number, NCI activity code

Standardize name of funder; classify funder type

Source

Journal Article

Presentation

FDA Press Release

List of acknowledgements/authors

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What is the role of NCI support in the development of cancer biomarkers?

- **Source:** Biomarkers Module of Thomson Reuters Integrity℠ – a manually curated database of biomarkers with standardized terminology classifying biomarkers into lifecycle phases and disciplines
- **Key data:** Piloted with breast cancer biomarkers
- **Basis:** specific PubMed journal articles mentioning biomarker
- **Linkage to NCI:** funding acknowledgements
Biomarkers

Thomson Integrity Manually-Curated Databases

Thomson Reuters ScienceWire Publication Catalog

PMID

NCI Funding

Literature references used as links to NCI funding

Biomarker

Indication

Use

Validity

References

Population Role (e.g., prognosis, diagnosis)

Technique

Substrate (e.g., plasma, tissue)

Early/Late Stage

Breast Cancer
Patent Analysis

What is the role of NCI support in FDA-approved drugs?

- **Source:** FDA Orange Book
- **Key data:** Identifies drug products approved on the basis of safety and effectiveness by the Food and Drug Administration (FDA)
- **Basis:** patents cited in New Drug Applications (NDAs), journal articles included in non-patent references, and patents cited by NDA-associated patents
- **Linkage to NCI:** Notices of Government Interest, Intramural Assignees, funding associated with non-patent references

http://www.fda.gov/Drugs/InformationOnDrugs/ucm129662.htm
What is the role of NCI support in guidelines for treatment, screening & detection, and supportive care?

- **Source:** The National Comprehensive Cancer Network® (NCCN®) Guidelines
- **Key data:** Guidelines developed by topic-specific expert panels
- **Basis:** cited references / publications
- **Linkage to NCI:** If references were journal articles, automated searches of NIH databases (SPIRES, MEDLINE) used to find NIH and NCI award numbers

Strengths & Limitations

Strengths:

- independent sources of key data
- most based on publicly available data
- relevance to different components/stages of research process
- scientific expert input

Limitations:

- not designed for program evaluation
- vary in level of effort and depth of analysis
- retrospective focus
- attribution/directness/meaning of links to funder sponsored projects
- mainly pilot analyses of limited scope
- some proprietary data sources
Future Directions

• Integrate methods shown to provide richer information on NCI impact
• Trace backward to underlying basic research
• Trace forward
  ▪ test accuracy of JCO expert predictions
  ▪ impact of clinical practice changes resulting from NCCN guidelines
  ▪ broader societal impacts such as improved public health or economic benefits (e.g., actual clinical practice by oncologists)
• Expand beyond NCI/NIH funding as additional funders acknowledge support in a standardized fashion
• Explore integrating information into analyses to support NCI decision making processes

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Nearly half of major advances and notable research acknowledged NCI support.
Sample Results: Biomarkers

Where in the pipeline are most of the biomarkers that were supported by NCI?

- Recommended / Approved: 13.2%
- Late Studies in Humans: 24.4%
- Early Studies in Humans: 13.1%
- Experimental: 18.5%
- Emerging: 11.5%

Which funding mechanism has made the largest contribution?

- Research Projects (R): 505
- Center Grants (P): 161
- Cooperative Grants (U): 105
- Research Career Programs (K): 46
- Training Programs (T): 25
- Intramural Research (Z): 18
- Contracts (N): 9
- Fellowship Programs (F): 7
- Loan Repayment Programs (L): 2

NCI intramural and contract awards may be underrepresented due to fewer links being available through the current automated approaches.
Sample Results: Patent Analysis

- 245 Drugs linked to 1,027 unique NCI projects
- 411 drug-project links had supporting data using the current framework
- Quick, transparent method to draw attention to higher confidence links

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<tr>
<th>Score</th>
<th>Number of Drug-Project Links</th>
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• NIH Scientific Management Review Board - Report on Approaches to Assess the Value of Biomedical Research Supported by NIH – March 2014

• NIH currently planning for launch of developing responses to recommendations

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